

We claim:

1. An isolated Gcc DNA molecule, wherein the DNA molecule has a modification in at least one nucleotide that disrupts a splicing consensus sequence and prevents splicing of mRNA produced from the DNA molecule, while preserving the ability of the DNA to express active Gcc.
2. The DNA molecule of claim 1, wherein the modification impairs a consensus nucleotide sequence needed to induce splicing.
3. The DNA molecule of claim 2, wherein the DNA molecule is modified at two cryptic splice sites.
4. The DNA molecule of claim 1 or 3, comprising a mutation in the 3' junction site.
5. The DNA molecule of claim 4, wherein the mutation is as shown in the 3' junction site in Table 1, or a functionally equivalent mutation.
6. The DNA molecule of claim 1 or 3, comprising a mutation in the 5' splice junction site
7. The DNA molecule of claim 6, wherein the mutation is as shown in the 5' junction site in Table 1, or a functionally equivalent mutation.
8. The DNA molecule of claim 1, comprising all or part of the nucleotide sequence shown in figure 4(b).
9. A vector comprising the DNA molecule of any of claims 1 to 8.
10. The vector of claim 9, comprising a promoter that is functional in a mammalian cell.
11. mRNA produced from the DNA molecule of any of claims 1 to 8 or the vector of claim 9 or claim 10.
12. A method of medical treatment of Gaucher disease in a mammal, comprising administering to the mammal an effective amount of the nucleic acid molecule of any of claims 1 to 8 or the vector of claim 9 or claim 10 and expressing an effective amount of the polypeptide encoded by the nucleic acid molecule for alleviating clinical symptoms of Gaucher disease.
13. A host cell, or progeny thereof, comprising the nucleic acid molecule of any of claims 1 to 8 or the vector of claim 9 or claim 10.

14. The host cell of claim 13, selected from the group consisting of a mammalian cell, a human cell and a Chinese Hamster Ovary cell.
15. A method for producing a recombinant host cell capable of expressing a Gcc nucleic acid molecule, the method comprising introducing into the host cell the vector of claim 9 or 10.
16. A method for expressing a Gcc polypeptide in the host cell of claim 13 or 14 comprising culturing the host cell under conditions suitable for DNA molecule expression.
17. A method for producing a transgenic cell that expresses elevated levels of Gcc polypeptide relative to a non-transgenic cell, comprising transforming a cell with the vector of claim 9 or 10.
18. An isolated polypeptide encoded by and/or produced from the nucleic acid molecule of any of claims 1 to 8, or the vector of claim 9 or 10.
19. A method of producing a genetically transformed cell which expresses or overexpresses a Gcc polypeptide, comprising:
 - (a) preparing a Gcc nucleic acid molecule according to any of claims 1-18;
 - (b) inserting the nucleic acid molecule in a vector so that the nucleic acid molecule is operably linked to a promoter;
 - (c) inserting the vector into a cell.
20. A transgenic cell produced according to the method of claim 19.
21. A pharmaceutical composition, comprising a carrier and (i) the nucleic acid molecule of any of claims 1 to 8 (ii) the vector of claims 9 or 10 or (iii) Gcc polypeptide produced from (i) or (ii), in an effective amount for reducing clinical symptoms of Gaucher disease.
22. The composition of claim 21, wherein the carrier comprises a liposome.